

GTx-001 Non-technical Abstract

The currently available therapies for many cancers are ineffective and many have severe, potentially fatal side effects. Specifically, locally advanced prostate cancer results in death of more than 75% of newly diagnosed patients. Gene replacement therapy is an attempt to treat the specific underlying cause of the cancer. Normal cells have very tightly controlled growth. Prostate cancer is the result of uncontrolled cell growth within the prostate gland. A gene known as p16 is one of many genes that act to control growth in normal cells. Several studies have shown that p16 is defective in a large number of prostate cancer cells. The novel therapy that we have studied extensively within the laboratory and in animal systems involves placement of a functioning copy of the p16 gene directly into prostate cancer cells. The delivery of the normal p16 gene is accomplished using a disabled virus that is normally responsible for the common cold. This type of damaged virus is called a vector. The vector functions as an efficient vehicle to place the new gene into the cancer cells but does not participate in the therapy itself. These vectors have proven to be safe in our laboratory studies and in prior trials that involved treatment of patients. Once the new p16 gene is in the prostate cancer cells, it may direct these cells to stop growing and possibly to die. Since our tests indicate that the new gene only remains with the cancer cells for a short period of time, multiple treatments are planned to potentially maximize the effectiveness of the new p16 gene. Finally, since this treatment specifically targets the prostate cells, we anticipate that there will be no serious adverse side effects associated with the therapy besides for the discomfort attributed the needle insertion into the prostate gland.